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A Health Economics Approach to US Value Assessment Frameworks: Summary and Recommendations of the ISPOR Special Task Force Report

Abstract

This summary paper first lists key points from each of the six sections, followed by six key recommendations. The Special Task Force chose take a health economics approach to the question of whether a health plan should cover and reimburse a specific technology, beginning with the view that the conventional cost per quality-adjusted life year (QALY) metric has both strengths as a starting point and recognized limitations. This report calls for the development of a more comprehensive economic evaluation that could include novel elements of value (e.g., insurance value, equity) either as part of an "augmented" cost-effectiveness analysis or a multi-criteria decision analysis. Given an aggregation of elements to a measure of value, consistent use of a cost-effectiveness threshold can help assure the maximization of health gain and well-being for a given budget. These decisions can benefit from the use of deliberative processes. The six recommendations are to: (I) Be explicit about decision context and perspective in value assessment frameworks; (II) Base health plan coverage and reimbursement decisions on an evaluation of the incremental costs and benefits of healthcare technologies as is provided by cost-effectiveness analysis; (III) Develop value thresholds to serve as one important input to help guide coverage and reimbursement decisions; (IV) Manage budget constraints and affordability based on cost-effectiveness principles; (V) Test and consider using structured deliberative processes for health plan coverage and reimbursement decisions; (VI) Explore and test novel elements of benefit to improve value measures that reflect the perspectives of both plan members and patients.

Preamble:

During the course of the STF's work, we invited external input on two earlier versions of draft reports. A first draft report was sent to the STF's External Advisory Board and Stakeholder Advisory Group on May 4, 2017. In response to feedback received, we posted a revised version to the full ISPOR membership on July 7, 2017.

These efforts resulted in many thoughtful and often detailed comments from a wide range of individuals representing diverse stakeholders, including patient organizations, payers, academic researchers, and the pharmaceutical and medical device industries. The STF greatly appreciates this input and the final report is much improved because of it. We begin this final section with a summary of what we heard and how we responded.

Some reviewers praised the tenor and scope of the report and recommendations. Some underlined generally the need for more emphasis on transparency and stakeholder input into value framework methods and processes.

Many reviewers offered constructive criticism. A number emphasized that the report should be more "patient-centric." Specifically, they emphasized that the patient perspective and patient voice needed to be reflected in all discussions about value—for example, that value measurement should consider patients' personal goals and preferences for different treatment options.

Some commenters highlighted the shortcomings of the quality-adjusted life year (QALY) metric, noting that QALYs often do not capture patient preferences well and potentially discount the value of an

individual's disability. Numerous reviewers addressed the STF's recommendations regarding the use of cost-per-QALY metrics to inform public and private decision making. Some spoke in favor of this recommendation, though others cautioned that such use could impede access to important new treatments, and, more generally, argued that any overarching STF recommendations calling for payers to apply cost-per-QALY analyses and cost-effectiveness thresholds was not consonant with the pluralistic, market-based U.S. health system. Some pointed out the US government's own restrictions on use of cost-per-QALY thresholds as evidence of the metric's limitations and public opposition.

The STF considered each comment carefully as we revised the report, mindful of the diverse membership of ISPOR and the organization's mission to advance good methods and informed decision making in pharmacoeconomics and outcomes research. Compared to earlier drafts, the final report contains more text on the importance of patient centricity, for example. In numerous places, we qualified language in response to feedback. We recognize that given the varied opinions of ISPOR members, not everyone will be satisfied with our judgments. Inviting the external commentaries that accompany the formal publication of this effort is one further attempt to ensure that diverse views are aired. The larger conversation about value metrics will undoubtedly continue on many fronts.

This summary section presents a list of key points from each of the six sections of our report, followed by a section listing our six key recommendations [1-6]. It is important to note that this report reviewed five recent US value assessment frameworks that differ by perspective and decision context. From a health economics perspective, the primary focus of our recommendations is on US payers—private and public. Broadly, our Special Task Force recommends greater use of cost-effectiveness analysis (CEA) in their decision making in order for them to best serve the interests of the plan members and patients who they represent. We also recognize, however, that there are challenges in applying CEA as well as a need for more research on the elements of value, on their aggregation, and on how they are used in deliberative decision making.

7.1 Section 1—Introduction [1]

- Concerns about rising prescription drug prices have led to initiatives in the U.S. designed to measure and communicate the value of pharmaceuticals.
- Organizations promulgating value assessment frameworks differ in their missions, activities, and approaches.
- This paper is based on the premise that value-based resource allocation decisions—about drugs and other healthcare technologies—should consider the full costs and benefits of decisions to relevant stakeholders and the decision contexts they face.
- We define "value" from an economic perspective in two related, but distinct senses: "gross value" is what individuals (or others acting on their behalf) would be willing to pay to acquire more health care or other goods or services. This has to be compared with the "opportunity cost" in terms of what benefits or other resources they are willing to forgo to obtain them. The difference between the two is the "net value."
- Rewarding healthcare technology manufacturers based on value is important because it sends signals to them that can influence R&D and ultimately innovation.
- Health economists have long recommended that analysts seeking to inform resource allocation decisions approximate an intervention's value in terms of incremental cost per QALYs gained (or the similar disability-adjusted life year (DALY) used commonly in global health evaluations).

- QALYs may not always fully capture the health (or well-being) of patients, or incorporate individual or community preferences about the weight to be given to health gain—e.g., about disease severity, equity of access, or unmet need.

7.2 Section 2— An Overview of Value, Perspective, and Decision Context [2]

- Because individuals vary in terms of their preferences for health care versus other goods, partly due to varying incomes, and in terms of their preferences for different health outcomes (e.g., survival vs. quality of life), for any specific health care technology, there would be a distribution of valuations in a population.
- Insurers—both private and public—act as agents on behalf of their enrollees who are potential patients by obtaining or providing access to healthcare technologies.
- Given that most medical care is purchased indirectly via health insurance, individuals do not directly face prices, and their agents (insurers and providers) acting on their behalf must assess value.
- CEA, by relating an intervention's cost to its effectiveness (In terms of some change in health) in a ratio is thus a standard approach to measuring the net value of a healthcare intervention.
- Existing guidelines for CEA emphasize the importance of clearly specifying the perspective from which the analysis is undertaken. Relevant perspectives may include among others: (i) the typical health plan enrollee; (ii) the patient; (iii) health plan manager; (iv) the provider; (v) the technology manufacturer; (vi) the specialty society; (vii) government regulators, or (viii) society as a whole. A valid and informative CEA could be conducted from the perspective of any of these stakeholders, depending on the purpose of the analysis.
- The five recent value frameworks that motivated this Special Task Force vary in the decisions that are being informed by the valuation, ranging from coverage, access, and pricing, to defining appropriate clinical pathways, and to supporting provider-clinician shared decision making.
- This economic concept of value does not depend on whether value is being measured within a market-based or a single-payer health care system. Health economics and outcomes researchers generally measure value using the tool of CEA with the QALY as the heath gain measure.
- Information about value is, however, used differently in different types of health care systems and by different stakeholders in different decision contexts.
- Payers may wish to use valuation by CEA to support coverage decisions as well as negotiations with manufacturers over a "value-based price." A health economics approach is most relevant to this decision context, and thus is the focus of this report.
- Specialty societies (such as ACC) or other organizations (such as NCCN or ASCO) may use CEA or other value frameworks to design appropriate clinical pathways or to support shared clinical decision making.
- Each of these value frameworks must be evaluated in its own decision context for its own objectives.
- In market-based systems, the individual—wearing the two hats of the patient and the plan enrollee (i.e., a potential patient)—tends to be the ultimate decision maker, but individuals make these decisions with the assistance of agents—the insurer and providers.
- CEA can be patient-centric in that valuation can consider the impact of specific disease attributes on patients' length of survival and quality of life.
- In a market-based system, decision making is more decentralized. Consumers making decisions about the purchase of private health insurance or out-of-pocket spending may vary in their objectives and preferences. Thus, they will choose among different health plans that will have different willingness to pay for QALYs and so different cost-effectiveness thresholds.

7.3 Section 3--Defining Elements of Value in Health Care [3]

- The underlying concept of value from a health economic perspective is typically measured using CEA. The cost-effectiveness of a medical technology is always calculated relative to alternative choices. For this reason, CEA focuses on *incremental* costs and *incremental* benefits.
- As recommended by the Second Panel, a wide range of costs or cost savings—present and future—should be considered, so long as they result directly from the interventions of interest.
- Benefit is measured from the perspective of the patient (or potential patient) given the healthcare technology in question. Ideally, we would have some way of capturing all these changes in terms of a common unit.
- To solve this problem, health economists have developed the concept of a "quality-adjusted life-year" (QALY), which can in principle be used to measure the health benefit of any technology, regardless of the disease it treats.
- QALYs and costs often form the basis of value assessments based on CEA. However, QALYs capture only a subset of benefits that may be produced by a health care intervention. This framework neglects numerous alternative aspects of benefits that should also be considered.

- Despite its challenges, the QALY remains the most accepted measure for capturing the incremental benefit of a treatment for use in population-level decision-making. However, some additional elements that may reflect value but are not normally captured in CEA with the QALY should be considered as well, depending on the perspective of the analysis.
- Productivity gains, net of consumption, should be included for societal-level perspectives, in the incremental cost calculations for CEA.
- Best practice for CEA measurement includes capturing the effects (on both outcomes and costs) of real world behavior such as adherence to an intervention.
- Some value elements, such as the value of knowing as part of diagnosis, or the psychic fear of contagion, are generally not captured but should be considered when relevant.
- Other value elements including value of insurance, severity of disease, value of hope, and real option value have been shown to modify QALY estimates, but are not commonly used in CEA Further research to evaluate their potential for more standard use is warranted.
- Two other potential value elements—equity (beyond severity effects discussed above) and scientific spillover effects—may have both individual and societal implications; more research on how to measure and incorporate these elements is also suggested.
- Augmenting CEA to consider these additional elements would result in a more comprehensive CEA in line with the Second Panel's Impact Inventory.
- More research and experience are needed in the application of augmented CEA (ACEA) and the Impact Inventory. To the extent that additional elements can be valued in monetary terms, the evaluation more closely resembles a CBA (cost-benefit analysis), which is sometimes termed "net monetary benefit" (NMB).

7. 4 Section 4—Objectives, Budgets, Thresholds and Opportunity Costs

- Use of value assessment frameworks for coverage and pricing decisions ideally requires a decision rule about what constitutes good value for money. Consistent use of a cost-effectiveness threshold, with potential consideration of other elements, can help assure the maximization of health gain (e.g., QALYs), for a given budget.
- The choice of a threshold hinges on the decision-maker's budget, given the distribution of health care technologies: the higher the threshold, the larger must be the budget to accommodate all technologies that meet that threshold. In a health care system with a fixed budget, in the short run, the threshold should reflect the opportunity cost of the technologies displaced when new technologies are introduced.
- Thresholds and budgets used to make coverage decisions from a payer perspective, should—to the extent possible—reflect consumers'/taxpayers' WTP for health and financial protection *ex ante,* i.e., preferences for insurance before individuals require health care.
- We do not recommend considering budget impact as an integral part of value assessment itself or structuring/requiring an automatic discount linked to budget impact, or introducing an inverse relationship between value and budget impact.
- Even where decision makers operate with an explicit threshold or threshold range, a deliberative process is generally followed to allow for external considerations that may modify the choice of threshold, such as equity or end-of-life concerns.

7. 5 Section 5—Approaches to Aggregation and Decision Making

- While conventional CEA and CBA are the gold standards for combining components of value and cost into a single metric, they have limitations in terms of how they address aggregation across individuals (incorporating equity, fairness, disparities, and other criteria), and in practice they do not incorporate some important components of value and cost.
- Extended CEA (ECEA) is a method for evaluating the distributional effects on financial risk of a medical intervention on a set of population subgroups of interest. However, by creating separate measures of health gain and financial risk, it does not provide a single metric for treatment evaluation.
- Augmented CEA (ACEA) is a proposed method for including novel elements of value in treatment evaluation. It may be expressed as a multi-dimensional inventory of effects, as a cost-effectiveness ratio with additional elements included in both cost and utility, or as net monetary benefit. More research and testing is needed before this approach can be incorporated in decision making.
- Public and private insurers use deliberative decision making for payer coverage and reimbursement decisions. This can be improved, both by the introduction of cost-per-QALY evidence and by the use of more structured decision making to take account of preferences about the weight to be given to health gain compared to other attributes such as disease severity, equity of access, or unmet need.
- A transparent deliberative process can also increase the legitimacy of decision making. Currently, processes often lack transparency, and it is unclear what factors have been considered and how decisions were reached. Deliberative processes today are often informal and unstructured. Thus, without a standardized approach, key issues may be overlooked, decisions may be reached in an unstandardized way, and potential biases of decision makers may not be adequately explored.
- Multi-criteria decision analysis (MCDA) provides a constructive approach to inform decision making about value, but its theory, application, and implementation need additional research.

Novel methods for acquiring value weights, particularly in settings with committees acting as decision-makers, may hold promise.

7.6 Section 6 - Review of Recent U.S. Value Frameworks

- The recently proposed US value frameworks can be characterized in terms of their decision contexts and perspectives, which determine how each accounts for costs and outcomes.
- The frameworks show substantial diversity in how they define and measure value, ranging from CEA to shared decision making by clinicians and patients.
- While the frameworks have contributed in important ways to debates about value, they have been criticized on various grounds, for example, lacking conceptual foundation, omitting key components of cost or outcome given their perspectives, and not correctly considering uncertainty.
- Recent frameworks also vary considerably in whether and how they handle decision thresholds, equity, and budget concerns; our report provides some suggestions in these areas.
- Ideally, plan-level decisions would also incorporate individual and patient heterogeneity in outcomes, for example through sub-group analysis; while recent plan-level frameworks discuss these considerations, room for improvement remains.
- Patient-oriented value frameworks can play a useful role within health plans by, for example informing patients about the relative benefits and costs of therapies given different copayments. Allowing consumer choice across plans can help accommodate heterogeneity in preferences.
- There are many potential areas of improvement in how value frameworks assess benefits, costs, and other factors relevant to decision-making. Further research, testing, and discussion with stakeholders are needed to refine and enable such improvements for use in US health care systems.

7. 7 Key Special Task Force Recommendations

Our ISPOR Special Task Force on Value Assessment Frameworks makes the following key recommendations:

Recommendation I: Be explicit about decision context and perspective in value assessment frameworks.

1. No single value assessment framework can simultaneously reflect multiple decision contexts and the perspectives of the patient, the health plan, or society as a whole. Thus, it is important for any framework to clearly articulate the value construct it represents and the perspective and decision context in which it is to be used, and to be well validated and reliable within that construct and context.
2. For societal and health plan resource allocation decisions, including coverage and reimbursement decisions, the perspective used should reflect, at a minimum, those who ultimately pay for care, including, for example, enrollees, employees, and taxpayers.
3. Well-designed patient-level frameworks can help guide shared decision making for treatment choices among the clinically appropriate options that have been approved for coverage, so that patients and their providers can consider and weight factors most relevant to patient preferences and constraints.

Recommendation II: Base health plan coverage and reimbursement decisions on an evaluation of the incremental costs and benefits of healthcare technologies as is provided by cost-effectiveness analysis.

1. A central tenet in economics is to compare incremental costs and benefits in decision-making. Cost-effectiveness analysis (CEA) and, in particular, cost-per-quality-adjusted life year (QALY) analysis have many demonstrated strengths—and some recognized limitations; they are well established in health economics and utilized by decision makers in health systems worldwide.
2. Value assessment frameworks that focus on health plan coverage and reimbursement decisions should consider cost-effectiveness analyses, as measured by cost per QALY, as a starting point to inform payer and policy maker deliberations. In many instances, the cost-per-QALY metric can serve well as the core component of these assessments.
3. Elements of costs and benefits not normally included in CEA that affect individual well-being (such as severity of illness, equity, and risk protection) may be relevant for some health plan decisions; however, more research is needed on how best to measure and include them in decision making.

Recommendation III: Develop value thresholds to serve as one important input to help guide coverage and reimbursement decisions.

1. Payers—as agents for plan funders and patients—should consider decision rules guided by what they consider good value for money given their opportunity cost and budget constraints. Consistent use of a value threshold, such as cost-per-QALY limits or ranges, with potential consideration of other elements, can help to achieve maximum health gain and individual well-being for the available resources.
2. In the pluralistic US healthcare system, different health plans could use different thresholds or decision rules across or within plans for different types of patients, reflecting the differing generosity of their budgets and implying different levels of access to technologies. Equity, severity of illness or disability, and other considerations may alter thresholds in certain situations.

Recommendation IV: Manage budget constraints and affordability based on cost-effectiveness principles.

1. Budget impact analysis should not be an integral part of value assessment per se, but can play a role in addressing budget constraints and affordability
2. Issues related to budget constraints and affordability of healthcare technology are most efficiently addressed in the short term by considering: (a) the adjustment costs of reducing spending on, or replacing, existing technologies; (b) the health and cost impact of delaying or staging implementation of new technologies; and (c) the cost-effectiveness ratios of existing technologies relative to that of the innovation.
3. Over time, the availability of cost-effective new technologies may alter the desired amount of health care spending.

Recommendation V: Test and consider using structured deliberative processes for health plan coverage and reimbursement decisions

1. No existing method completely or perfectly solves the problems of aggregating cost and benefit information across individuals to a population level for benefit plan decision making or of combining multiple elements of value into a single value metric for individuals. Thus, pragmatic approaches are needed.
2. Certain health care decisions, such as decisions to cover and pay for new technologies, can benefit from using deliberative processes that consider CEA along with other relevant decision criteria.

3. Deliberative processes for value assessment should consider using explicit frameworks such as augmented cost-effectiveness analysis (ACEA) and multi-criteria decision analysis (MCDA) that emphasize structure and transparency.
4. MCDA models using individual patient-specific value weights could be developed to help patients choose among available medical interventions—those chosen for coverage by their health plan—using both their own value weights and their out-of-pocket costs.

Recommendation VI: Explore and test novel elements of benefit to improve value measures that reflect the perspectives of both plan members and patients.

1. We encourage development of a more comprehensive economic evaluation that could include novel elements of value—such as insurance value, real option value, value of hope, scientific spillovers, and others—and could ultimately provide for more efficient resource allocation within the health sector and between health and non-health spending. More research is needed on measuring the additional value provided by—and the willingness to pay for—these novel elements.
2. More research is needed on MCDA development and use, particularly for generating value weights and thresholds, as compared to other approaches. Alternative approaches for estimating value weights and thresholds in MCDA should be tested and compared both for methodological soundness and practical implementation factors (e.g., ease of use and reliability). Testing of MCDA models should include comparisons of their resource allocation implications with those of conventional or augmented CEA-based decision making as well as those of other decision approaches.
3. Payers serve as agents for their plan members who both pay premiums and become patients. Obtaining input on the value of health benefits from members of health plans, informed by patient experience, is central to the validity of value measures.

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